myeloablative auto or allo-HCT with or without comorbidities (n = 15). Median HCT-CI score was 2 (range, 0-5) (n = 25). Pts were conditioned with either 2 Gy TBI (n = 3), or Flu 90 mg/m^2 + 2 Gy TBI (n = 23). Pts were given peripheral blood stem cells (PBSC) (n = 24) or marrow (n = 2) grafts from HLA-identical sibs or unrelated donors matched for 10/10 (n = 15), 9/10 (n = 5), or 8/10 (n = 1) alleles. Post-grafting immunosuppression consisted of either CSP/MMF (n = 22), tacrolimus/MMF (n = 1), or tacrolimus/MMF/sirolimus (n = 3). Donor CD3 chimerism was documented in 25 pts at day 28, but was not evaluated in 1 pt who died on day 23 after achieving normal granulocyte counts. Secondary graft rejection occurred at day 56 in 1 pt with MDS given marrow, who subsequently achieved full donor chimerism after a second myeloablative HCT with PBSC. Of 24 evaluable pts, 20 converted to full donor CD3 chimerism (95-100%) while 4 remained with mixed CD3 chimerism (15-93%). Twenty pts developed acute GVHD (grade II = 17; III = 3) at a median of 34 (range, 9-56) days after HCT. The cumulative incidence of chronic GVHD was 51% at 2 yrs. Causes of death included disease progression/relapse (n = 13), complications of GVHD (n = 4), or sepsis/pulmonary complications (n = 2). The 2 year overall survival (OS), progression-free survival, relapse, and non-relapse mortality were 49%, 28%, 58% and 14%, respectively. On univariate analysis, pts with HCT-CI ≥3 had worse OS (p = 0.008). In this small cohort, there was no survival benefit for pts with myeloid vs lymphoid malignancies or disease status at HCT. In conclusion, NM-HCT remains an important curative option for a subset of high-risk pediatric pts who would otherwise not qualify for high-dose therapy, with relapse being the highest contributor to overall mortality.

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HIGH-DOSE CHEMOTHERAPY WITH AUTOLOGOUS STEM-CELL RESCUE IN PEDIATRIC PATIENTS WITH CNS TUMORS: TOXICITY AND OUTCOMES

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We report the outcomes of all pediatric patients who received high-dose chemotherapy with autologous stem cell rescue for craniospinal (CNS) tumors at our institution. Thirty-two patients underwent 38 autologous transplants between 2002 and 2009. The population included 15 males and 17 females with a median age of 66 months at diagnosis and 87 months at transplant. The underlying diseases were medulloblastoma (56%), PNET (25%), ATRT (13%), and pinealoblastoma (6%). Thirty-seven percent of patients were treated for high-risk disease upfront, whereas 63% were treated for recurrent or progressive disease. The median pretransplant GFR was 119 ml/min/1.73 and 62% had hearing loss. No patients had significant pre-HSCT cardiopulmonary dysfunction. Conditioning regimens included carboplatin/thiotepa/etoposide (61%), carboplatin/thiotepa (21%), carboplatin/thiotepa/VP16 (5%), thiotepa/etoposide (5%), and other (8%). Of those that received a carboplatin-based regimen, 11% had dose-reduction due to pre-existing organ toxicity. Median time to engraftment was 11 days. Of the 97% of patients who survived to discharge, the median duration of hospitalization was 20 days. Acute complications included infection (37%), hypertension (16%), VOD (13%), respiratory compromise (13%), renal failure (11%), and CNS hemorrhage (8%). Four patients (11%) required ICU care. Late complications included transverse myelitis (2 patients), varicella zoster (2 patients), and esophageal stricture (2 patients). Progressive hearing loss was reported in 9 patients. The median duration of follow-up was 502 days. The overall survival was 72% and progression-free survival was 56%. All deaths after discharge were due to progressive disease. Of the 12 patients who were transplanted upfront, 11 (92%) are alive without disease progression and 1 patient died of recurrent disease (median duration of follow-up 1290 days). Of the 20 patients transplanted for recurrent disease, 1 died during transplant, 6 (30%) are alive without progression, 6 (30%) are alive with progression, and 7 (35%) are dead due to recurrent disease (median duration of follow-up 358 days). Autologous stem cell transplant is well-tolerated with little significant toxicity in this population. Pediatric patients with CNS tumors and high-risk disease who are treated upfront appear to have better disease-free survival than patients with recurrent disease who undergo autologous transplant.

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RESULTS OF A SURVEY OF HEMATOPOIETIC STEM CELL TRANSPLANTATION: RESULTS IN BRAZILIAN PATIENTS ≤ 20 YEARS WITH ACUTE LYMPHOBLASTIC LEUKEMIA

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The Brazilian Society of Pediatric Oncology (SOBOPE) is in the process of developing a new national protocol for the treatment of acute lymphoblastic leukemia (ALL). Since all transplant indications shall balance risks and benefits of each treatment modality, we would have to know the results of hematopoietic stem cell transplants (HSCT) performed in our country. Therefore, we analyzed the pediatric data reported in a voluntary national survey of all transplants performed for the treatment of ALL.

Methods: A questionnaire with demographic data and transplant results was sent to all Brazilian HSCT units to get data on all consecutive patients transplanted for ALL. The 20 years of age cut of was chosen to allow comparison with CIBMTR data.

Results: Twenty institutions sent their data on 532 patients ≤ 20 years. Median age was 10 years (0.5-29), 66% male. The median interval from diagnosis to transplant was 2.5 years (1 mo-19 y) and median follow-up, 3 years (9 mo-14 y). Most patients were transplanted in second remission (CR2 – 43%), followed by advanced phases (10% CR3 and 26% in relapse) and only 19% in CR1. Matched sibling donors transplant were 74% (N = 374), with a 35% 3-year overall survival (OS) and event free survival (EFS). HSCT results have significantly improved over time from 27% in the early 90's to 46% 2005-2009 (Log rank p <0.001), in a shorter follow-up. In related donor HSCT, age, conditioning therapy (+/- TBI) and graft source did not influence survival, but EFS was 61% in CR1, 36% in CR2 and 22% in more advanced phases (p < 0.0001). Related and unrelated donor transplants had the same results (with no adjustment). Unrelated transplants were performed in 131 patients. Age < 10 years (p = 0.004), lower disease risk (p = 0.02), the use of cord blood (p = 0.01), and TBI (p = 0.001) were associated with a better outcome. Unrelated donor transplants had high number of early toxic deaths and 3-yr EFS of 46% in CR1. Compared with CIBMTR data, related donor transplants have similar results (3-yr EFS 61% in CR1).

In conclusion, one third of our patients had HSCT for ALL in advanced stages. Results have improved in the past years for related transplants. Disease stage was one of the most important risk factors. After unrelated transplants, age <10 years and the use of cord blood had better outcomes. These results will contribute to the design of the new pediatric ALL Brazilian protocol.